



**[Billing Code 4140-01-P]**

**DEPARTMENT OF HEALTH AND HUMAN SERVICES**

**National Institutes of Health**

Prospective Grant of Exclusive Patent License: Capsid-Free AAV Vectors, Compositions, and Methods for Vector Production and Gene Delivery

**AGENCY:** National Institutes of Health, HHS.

**ACTION:** Notice.

**SUMMARY:** The National Heart, Lung and Blood Institute (NHLBI), National Institutes of Health, Department of Health and Human Services, is contemplating the grant of an exclusive patent license to Generation Bio Co. (“Generation Bio”), a company based in Cambridge, Massachusetts (in the exclusive field specified below), and a co-exclusive license to Generation Bio and Spark Therapeutics, a company based in Philadelphia, Pennsylvania (in the co-exclusive field specified below), to practice the inventions embodied in the patent application listed in the Supplementary Information section of this notice.

**DATES:** Only written comments and/or applications for a license which are received by the NHLBI Office of Technology Transfer and Development within 15 days from the date of publication of this notice in the Federal Register will be considered.

**ADDRESSES:** Requests for copies of the patent applications, inquiries, and comments relating to the contemplated exclusive patent license should be directed to: Uri Reichman, Ph.D., MBA, Senior Licensing and Patenting Manager, 31 Center Drive Room 4A29, MSC2479, Bethesda, MD 20892-2479, phone number 301-435-4616, or [uri.reichman@nih.gov](mailto:uri.reichman@nih.gov)

**SUPPLEMENTARY INFORMATION:** The following and all continuing U.S. and foreign

patents/patent applications thereof are included in the intellectual property to be licensed under the prospective agreements to Generation Bio and Spark Therapeutics: NIH reference # E-241-2010.

US patent 9,598,703 issued March 03, 2017; Israeli patent 228328 issued December 01, 2018; Australian patent 2012228376 issued October 05, 2017, and pending applications in Brazil (BR 11 2013 023185 8 A2), Canada (application 2829518), China (application 201280022523.5), Europe (application 12 708035.6), India (application 8000/DELNP/2013), Japan (application 2013-557138), and S. Korea (application 10-2013-7026982).

The invention is jointly owned by the Government of the United States and by the following French institutions: Association Institut De Myologie, Sorbonne University, INSERM, and CNRS. The patent rights in these inventions have been assigned to the Government of the United States of America, and to the French institutions by their respective employees who are the inventors of the subject matter claimed in the patent rights. The prospective patent license will be granted worldwide and in fields of use not broader than the following:

Exclusive field: Electroporation-mediated delivery of DNA-based vectors to express therapeutic molecules for the treatment or prevention of human diseases.

Co-exclusive field: The treatment or prevention of cancer by administration of DNA-based vectors (with the exception of electroporation mediation) to express therapeutic molecules.

All Fields of Use with the exception of the aforementioned fields are available for licensing by other parties on nonexclusive terms.

The subject technology provides DNA-based constructs for human therapeutics or preventative therapies. Such DNA-based constructs may be useful in gene therapy for treating genetic disorders, or other diseases by expressing therapeutic molecules. These constructs are AAV genome-based, where the gene of interest (therapeutic payload) is inserted between two ITRs (Inverted Terminal Repeats). The resulting constructs are devoid of the AAV capsid, and thus nonviral. They are advantageous over conventionally used AAV vectors, as they are non-immunogenic. They are also advantageous over plasmid-based expression constructs since they are of eukaryotic origin and thus devoid of the bacterial-type DNA methylation as typically present in plasmids.

This notice is made in accordance with 35 U.S.C. 209 and 37 CFR Part 404. The prospective exclusive patent license will be royalty bearing and may be granted unless within fifteen (15) days from the date of this published notice, the NHLBI receives written evidence and argument that establishes that the grant of the license would not be consistent with the requirements of 35 U.S.C. 209 and 37 CFR Part 404.

Complete applications for a license in the prospective field of use that are timely filed in response to this notice will be treated as objections to the grant of the contemplated exclusive patent license.

Comments and objections submitted to this notice will not be made available for public inspection and, to the extent permitted by law, will not be released under the *Freedom of Information Act*, 5 U.S.C. 552.

Dated: September 17 , 2019.

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Uri Reichman Sr.,  
Senior Licensing and Patenting Manager,  
National Heart, Lung, and Blood Institute,  
Office of Technology Transfer and Development.

[FR Doc. 2019-20992 Filed: 9/26/2019 8:45 am; Publication Date: 9/27/2019]